

In Vivo Efficacy Fabry Mice: "Combination Therapy"

Pre-treat with single
infusion of α -galactosidase A

GB3 ↓

2 WEEKS

NB-DNJ, AMP-DNJ
or D-tet-P4
(IP dose, 1x daily)

4 WEEKS

GB3 ↑

Study Goal: Evaluate the effect of Substrate Inhibition
in Reducing GB3 Re-accumulation.

FIG. 1A

Fabry Mouse Liver

Re-accumulation of GB3 (IP delivery)

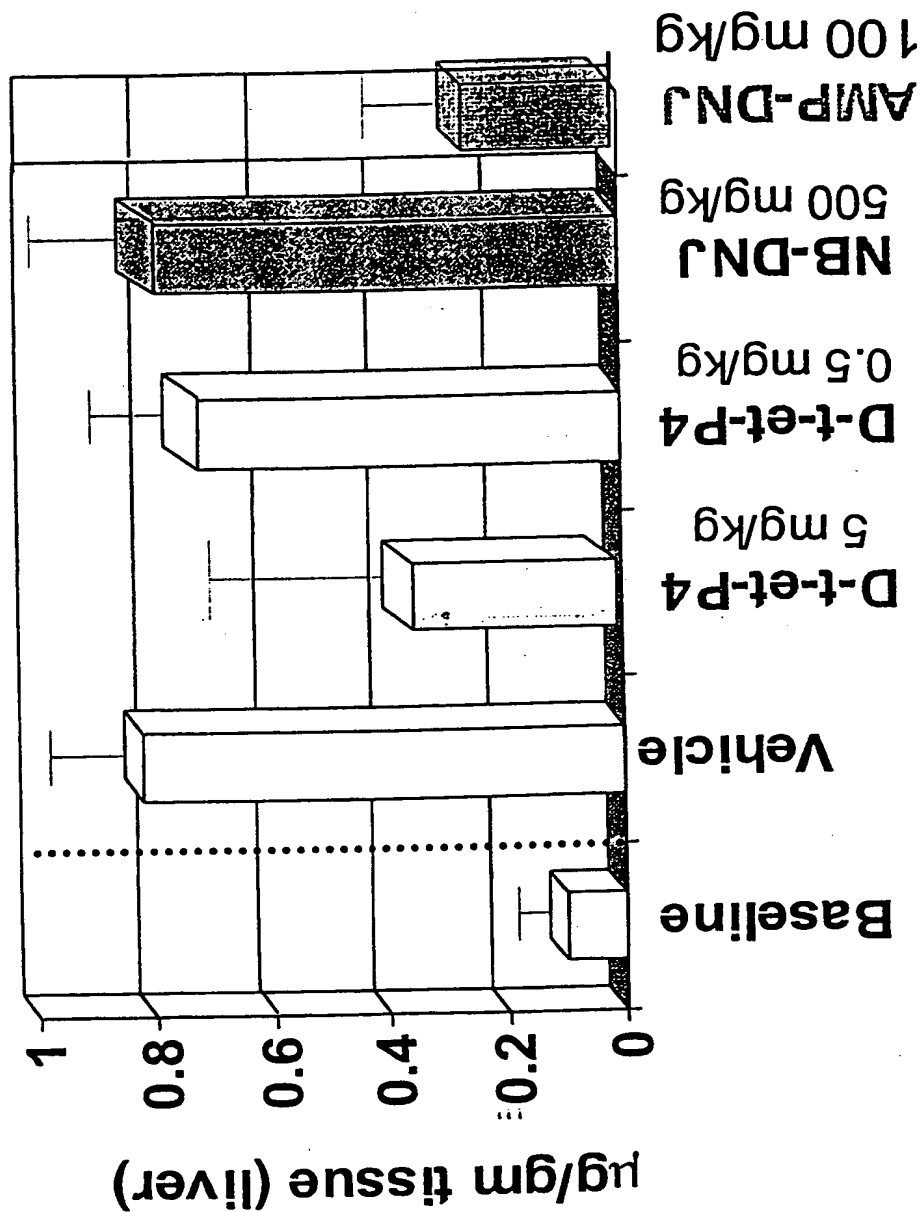


FIG. 1B